



## Ocugen Granted FDA Orphan Drug Designation for OCU400 (NR2E3) Gene Therapy for the Treatment of CEP290 Mutation Associated Retinal Disease

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MALVERN, Pa., Sept. 18, 2019 (GLOBE NEWSWIRE) -- [Ocugen, Inc.](#), a clinical stage biopharmaceutical company focused on discovering, developing and commercializing a pipeline of innovative therapies that address rare and underserved eye diseases, today announced the U.S. Food and Drug Administration (FDA) granted the second orphan drug designation for OCU400, Ocugen's novel gene therapy, for the treatment of *CEP290* mutation associated retinal disease. Inherited retinal diseases (IRDs) are caused by genetic mutations that are passed down within families and lead to visual impairment and blindness. Mutations in *CEP290* have been associated with different diseases including Leber Congenital Amaurosis (LCA), Bardet-Biedl syndrome, Joubert syndrome, Senior-Loken syndrome and Meckel-Grüber syndrome.

"The nuclear hormone receptor gene, *NR2E3*, plays a major role in modulating numerous biological pathways that are essential for maintaining the healthy status of the retina, both anatomically and functionally. In multiple preclinical animal models carrying defects in different genes that cause retinal dystrophy, *NR2E3* demonstrated its ability as a modifier gene to preserve retinal structure and function. We believe OCU400 has great potential to improve vision in patients with IRDs who harbor defects in unrelated genes other than *NR2E3*, by supplying the elevated level of *NR2E3* through a gene delivery approach," said Dr. Mohamed Genead, ophthalmologist/retina specialist and inherited retinal degeneration expert and co-founder, president of Aviceda Therapeutics LLC.

"We are very pleased to receive our second orphan drug designation for a gene therapy product from the FDA," said Shankar Musunuri, PhD, MBA, Chairman, Chief Executive Officer and Co-Founder of Ocugen. "Orphan designation for this indication supports the goal of our Modifier Gene Therapy Platform to treat a variety of inherited retinal diseases with a single gene therapy. There are currently no approved therapies for patients with the *CEP290* mutation, which leads to severe visual impairment that tends to progress slowly over time. There is a significant unmet medical need for these patients and an opportunity to improve their quality of life with this novel approach."

The FDA Office of Orphan Products Development grants orphan designation for novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. Orphan designation qualifies the sponsor of the drug for various development incentives of the Orphan Drug Act, including a seven-year period of U.S. marketing exclusivity, tax credits for clinical research costs, clinical research trial design assistance, the ability to apply for annual grant funding and waiver of Prescription Drug User Fee Act filing fees.

### About OCU400

OCU400 is a novel gene therapy with the potential to be broadly effective restoring retinal integrity and function across a range of genetically diverse IRDs. OCU400 is the first program that Ocugen is advancing based on its novel Modifier Gene Therapy Platform, developed by Neena Haider, PhD, Associate Professor of Ophthalmology at Harvard Medical School and Associate Scientist at the Schepens Eye Research Institute of Massachusetts Eye and Ear, from which Ocugen obtained an exclusive world-wide license to develop and commercialize ophthalmology products based on the platform. Consisting of a functional copy of the nuclear hormone receptor (NHR) gene *NR2E3*, OCU400 is delivered to target cells in the retina using an adeno-associated viral (AAV) vector. As a potent modifier gene, expression of *NR2E3* within the retina may help reset retinal homeostasis, stabilizing cells and potentially rescuing photoreceptor degeneration.

### About Ocugen, Inc.

Ocugen, Inc. is a clinical stage biopharmaceutical company focused on discovering, developing and commercializing a pipeline of innovative therapies that address rare and underserved eye diseases. The Company offers a robust and diversified ophthalmology portfolio that includes novel gene therapies, biologics, and small molecules and targets a broad range of high-need retinal and ocular surface diseases. Ocugen is leveraging its groundbreaking modifier gene therapy platform to address genetically diverse inherited retinal disorders (IRDs), including its therapies based on nuclear hormone receptor genes *NR2E3* (OCU400) and *RORA* (OCU410). OCU400 has received two orphan drug designations (ODD) targeting two distinct IRDs. Ocugen is also developing novel biologic therapies for wet-AMD, DME and diabetic retinopathy (OCU200), as well as for retinitis pigmentosa (OCU100). The Company's late-stage Phase 3 trial for patients with ocular graft versus host disease (oGVHD)(OCU300) leverages Ocugen's patented OcuNanoE – Ocugen's ONE Platform™ technology to enhance the efficacy of topical ophthalmic therapeutics. OCU300 is the first and only therapeutic with ODD for oGVHD, providing certain regulatory and economic benefits. For more information, please visit [www.ocugen.com](http://www.ocugen.com).

### Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. We may, in some cases, use terms such as "predicts," "believes," "potential," "proposed," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from the Company's current expectations. Any forward-looking statements that the Company makes in this press release speak only as of the date of this press release. The Company assumes no obligation to update forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

Contact:  
Ocugen, Inc.  
Kelly Beck

+1 484-328-4698